



EXHIBIT A

PENDING CLAIMS IN APPLICATION SERIAL NO: 08/475,470 PENNIE & EDMONDS DOCKET NO: 7639-077

1. A recombinant adeno-associated virus vector, which comprises:
 - a) at least a portion of the adeno-associated virus genome; and
 - b) at least one eukaryotic based nucleic acid sequence that encodes a wild-type gene product controlled by a eukaryotic based *cis*-acting regulatory sequence heterologous to the wild-type gene product, said virus vector having the property of regulating cell specific expression of said nucleic acid sequence or nucleic acid sequences upon stable transduction of a target mammalian cell.
4. A recombinant adeno-associated virus vector of Claim 1 wherein the mammalian cell is a human immune cell.
7. A recombinant adeno-associated virus vector of Claim 4 wherein said eukaryotic *cis*-acting regulatory sequence is chosen from the region located from about hypersensitive site I to about hypersensitive site IV, in association with the human globin gene cluster.
8. A recombinant adeno-associated virus vector of Claim 7 wherein said eukaryotic *cis*-acting regulatory sequence is chosen from the region located within the group of *cis*-acting regulatory sequences consisting of hypersensitive site I, hypersensitive site II, hypersensitive site III, hypersensitive site IV, and hypersensitive site VI, in association with the human globin gene.
9. A recombinant adeno-associated virus vector of Claim 4 wherein said nucleic acid sequence or nucleic acid sequences encodes at least one human gene protein, chosen from the human globin gene cluster.
10. A recombinant adeno-associated virus vector of Claim 48 wherein said eukaryotic *cis*-acting regulatory sequence is chosen from the region located from about

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hypersensitive site I to about hypersensitive site VI, in association with the human globin gene cluster.

11. A recombinant adeno-associated virus vector of Claim 10 wherein said eukaryotic *cis*-acting regulatory element is chosen from the region located within the group of *cis*-acting regulatory elements consisting of hypersensitive site I, hypersensitive site II, hypersensitive site III, hypersensitive site IV, and hypersensitive site VI, in association with the human globin gene cluster.

12. A recombinant adeno-associated virus vector of claim 48 wherein said nucleic acid sequence or nucleic acid sequences encodes at least one human gene protein, chosen from the human globin gene cluster.

16. A recombinant adeno-associated virus vector of Claim 4 wherein said *cis*-acting regulatory sequence comprises hypersensitive site II, associated with the human globin gene cluster.

17. A recombinant adeno-associated virus vector of Claim 4 wherein said nucleic acid sequence encodes a human globin protein [^]gamma globin.

19. A recombinant adeno-associated virus vector of Claim 4 wherein said immune cell is chosen from the group consisting of a human hematopoietic stem cell, a human myeloid progenitor cell and a human erythroid progenitor cell.

20. A recombinant adeno-associated virus vector of Claim 9 wherein said target immune cell is K562.

21. A recombinant adeno-associated virus vector of Claim 48 wherein said mammalian cell is an immune cell chosen from the group consisting of a human hematopoietic stem cell, a human myeloid progenitor cell and a human erythroid progenitor cell.



25. A recombinant adeno-associated virus vector of Claim 17 wherein said target immune cell is chosen from the group consisting of a human hematopoietic stem cell, a human myeloid progenitor cell and a human erythroid progenitor cell.

26. A recombinant adeno-associated vector of Claim 17 wherein said target immune cell is K562.

27. A recombinant adeno-associated virus vector, which comprises:

- a) at least a portion of the adeno-associated virus genome; and
- b) a eukaryotic based nucleic acid sequence that encodes a wild-type gene product controlled by a eukaryotic based *cis*-acting regulatory sequence heterologous to the wild-type gene product,

said virus vector having the property of regulatory cell specific expression of said nucleic acid sequence or nucleic acid sequences upon stable transduction of a primary human hematopoietic cell.

28. A recombinant adeno-associated virus vector of Claim 27 wherein said eukaryotic *cis*-acting regulatory sequence is chosen from the region located from about hypersensitive site I to about hypersensitive site VI, in association with the human globin gene cluster.

29. A recombinant adeno-associated virus vector of Claim 28 wherein said eukaryotic *cis*-acting regulatory element is chosen from the region located within the group of *cis*-acting regulatory elements consisting of hypersensitive site I, hypersensitive site II, hypersensitive site III, hypersensitive site IV, and hypersensitive site VI, in association with the human globin gene cluster.

30. A recombinant adeno-associated virus vector of Claim 29 wherein said *cis*-acting regulatory sequence comprises hypersensitive site IV, hypersensitive site III and hypersensitive site III.

31. A recombinant adeno-associated virus vector of Claim 27 wherein said nucleic acid sequence encodes a human globin protein, chosen from the human globin gene cluster.

33. A recombinant adeno-associated virus vector of Claim 27 which comprises a DNA sequence encoding a wild-type Fanconi anemia C complementing protein.

39. A recombinant adeno-associated virus vector of Claim 27 which comprises a DNA sequence encoding a wild-type Factor IX protein.

46. The recombinant adeno-associated virus vector of Claim 1 in which the portion of the adeno-associated virus genome comprises at least those nucleotide sequences encoding the inverted terminal repeats.

47. The adeno-associated virus vector of Claim 27 in which the portion of the adeno-associated virus genome comprises at least those nucleotide sequences encoding the inverted terminal repeats.

48. A recombinant adeno-associated virus vector comprising:

- a) at least a portion of the adeno-associated virus genome;
- b) a eukaryotic based nucleic acid sequence that encodes a wild-type gene product controlled by a eukaryotic based *cis*-acting regulatory sequence; and
- c) lacking a selectable marker,

said virus vector having the property of regulatory cell specific expression of said nucleic acid sequence or nucleic acid sequences upon stable transduction of a mammalian cell.

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